

Clinical Trial Targeting Lung Cancer, Plus Promising Osteoporosis and Incontinence Research get Support from Stem Cell Agency

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Oakland, CA – The five-year survival rate for people diagnosed with the most advanced stage of non-small cell lung cancer (NSCLC) is between one and 10 percent. To address this devastating condition, the Board of the California Institute for Regenerative Medicine (CIRM) today voted to invest almost \$12 million in a team from UCLA that is pioneering a combination therapy for NSCLC.

The team is using the patient's own immune system where their dendritic cells – key cells in our immune system – are genetically modified to boost their ability to stimulate their native T cells - a type of white blood cell - to destroy cancer cells. The investigators will combine this cell therapy with the FDA-approved therapy pembrolizumab (better known as Keytruda) a therapeutic that renders cancer cells more susceptible to clearance by the immune system.

"Lung cancer is a leading cause of cancer death for men and women, leading to 150,000 deaths each year and there is clearly a need for new and more effective treatments," says Maria T. Millan, M.D., the President and CEO of CIRM. "We are pleased to support this program that is exploring a combination immunotherapy with gene modified cell and antibody for one of the most extreme forms of lung cancer."

The CIRM Board also approved investing \$14.15 million in four projects under its Translation Research Program. The goal of these awards is to support promising stem cell research and help it move out of the laboratory and into clinical trials in people.

Researchers at Stanford were awarded almost \$6 million to help develop a treatment for urinary incontinence (UI). Despite being one of the most common indications for surgery in women, one third of elderly women continue to suffer from debilitating urinary incontinence because they are not candidates for surgery or because surgery fails to address their condition.

The Stanford team is developing an approach using the patient's own cells to create smooth muscle cells that can replace those lost in UI. If this approach is successful, it provides a proof of concept for replacement of smooth muscle cells that could potentially address other conditions in the urinary tract and in the digestive tract.

Max BioPharma Inc. was awarded almost \$1.7 million to test a therapy that targets stem cells in the skeleton, creating new bone forming cells and blocking the destruction of bone cells caused by osteoporosis.

In its application the company stressed the benefit this could have for California's diverse population stating: "Our program has the potential to have a significant positive impact on the lives of patients with osteoporosis, especially in California where its unique demographics make it particularly vulnerable. Latinos are 31% more likely to have osteoporosis than Caucasians, and California has the largest Latino population in the US, accounting for 39% of its population."

The successful applications are:

Application	Title	Institution	CIRM funding
TRAN1-10958	Autologous iPSC-derived smooth muscle cell therapy for treatment of urinary incontinence	Stanford University	\$5,977,155

TRAN2-10990	Development of a noninvasive prenatal test for beta-hemoglobinopathies for earlier stem cell therapeutic interventions.	Children's Hospital Oakland Research Institute	\$1,721,606
TRAN1-10937	Therapeutic development of an oxysterol with bone anabolic and anti-resorptive properties for intervention in osteoporosis	MAX BioPharma Inc.	\$1,689,855
TRAN1-10995	Morphological and functional integration of stem cell derived retina organoid sheets into degenerating retina models	UC Irvine	\$4,769,039

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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